Presentation to the Accessibility and Affordability Working Group

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CIRM
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CIRM
CALIFORNIA'S STEM CELL AGENCY

CIRM: Created by Prop 71 and continuing under Prop 14

Proposition 71
2004

$3B Bond Funding

Proposition 14
2020

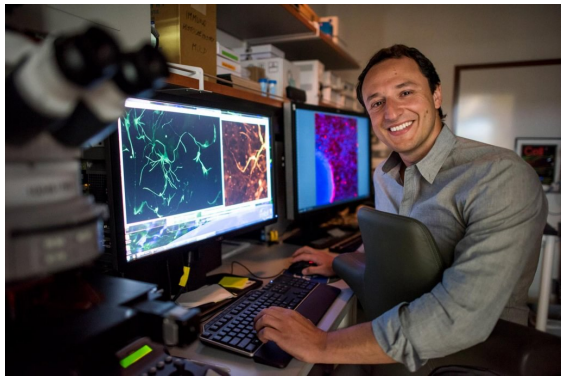
$5.5B Bond Funding

CIRM | Our Identity and Impact

- Funded **>1000 projects**

- Funded **76 clinical trials with 2700+ enrolled**
  - Across >35 disease, rare to common indications, from early stage to pivotal/phase 3 clinical trials
  - Furthest along to BLA: 6 gene therapies for monogenic disease, 1 CAR T, 4 cell-based therapies
  - Cures attained in clinical trial recipients in ADA SCID (50 patients 2-10 years out) and X-CGD

CIRM | Our Identity and Impact



- Generated **3,000 peer-reviewed publications** describing scientific and medical discoveries
- Funded **12 stem cell research facilities** and **17 shared research laboratories**
- Built the **largest iPSC research bank** with >2600 cell lines for modeling
- Enabled **invention of research and translational tools**
- Created **novel genomic datasets and bioinformatics tools for stem cell research**

CIRM | Our Identity and Impact

- Trained over **3,000 students and scholars** to become the future workforce of regenerative medicine
- Stimulated the CA economy with **$10.7B of gross output and 56,000 new FTE jobs** created during the 2004-2018 period
- Helped enable CIRM-funded projects to attract **$18B+ of industry funding**

CIRM | Evolving our Mission Statement

Real Life™

From

Accelerating stem cell treatments to patients with unmet medical needs.

To

Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world

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Strategic Plan for the Next Era of CIRM

Advance

World Class Science

Leverage collective scientific knowledge and an explosion of data through the creation of collaborative pathways

- competency hubs
- knowledge networks

Deliver

Real World Solutions

Advance more therapies to FDA marketing approval through partnerships with regulatory bodies, industry and academic and community medical centers.

- Leverage therapeutics development portfolio approach
- Fund a Manufacturing network
- Expand Alpha Clinics Network
- Develop Community Care Centers

Provide

Opportunity for All

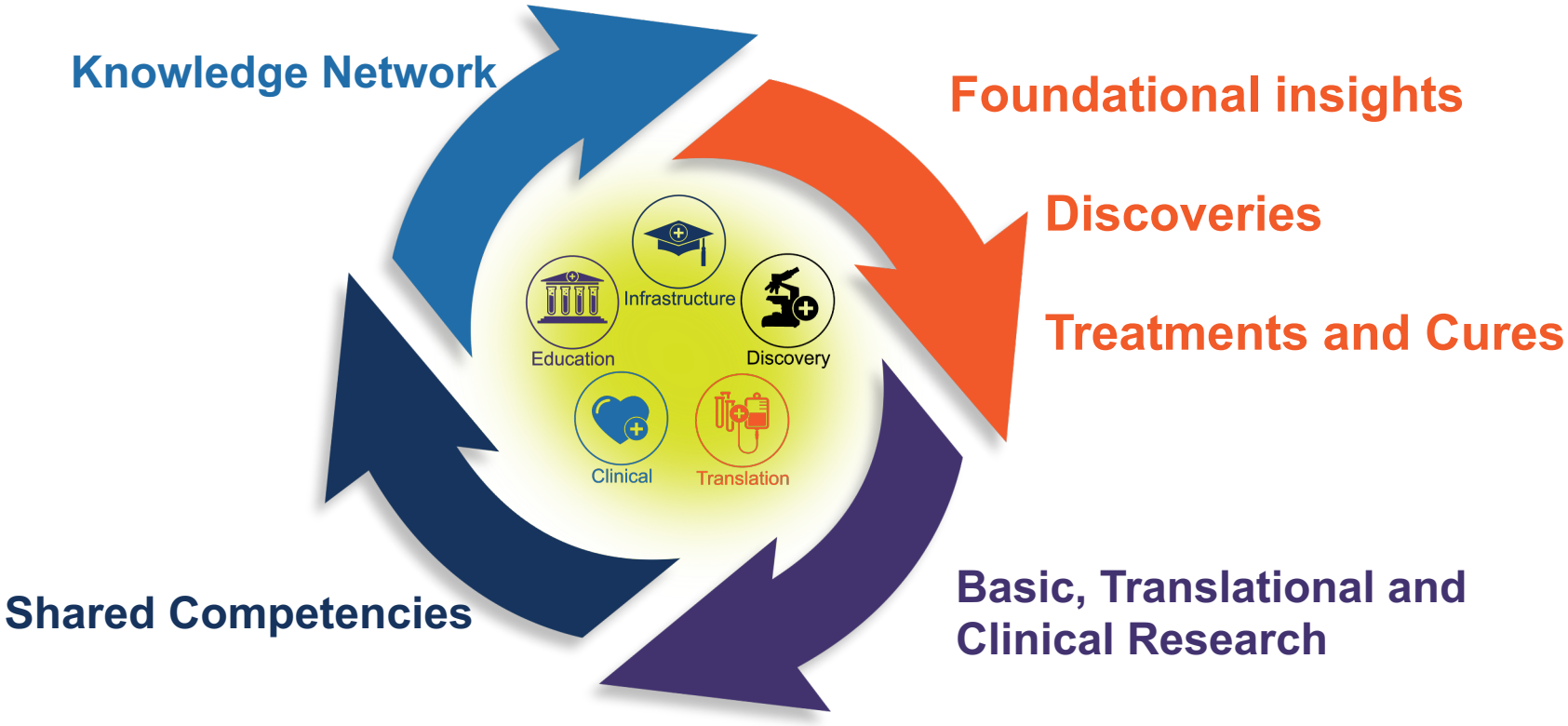
Build a diverse and highly skilled workforce and deliver accessible treatments to a diverse patient community

- EDU programs
- Roadmap for Access and Affordability

CIRM | Advance Word-Class Science

Real Life™

Create a systemic approach to foster a culture of collaboration and data sharing that would enable a stronger "evidence base" --- benefits science, product development and output may support coverage discussions



Deliver Real World Solutions

- Deploy CIRM's integrated therapeutics development program and partnership model to advance more therapies to FDA marketing approval
  - Well suited for portfolio-level discussions with FDA, payors and other stakeholders
  - Allows for expansion of CIRM's established "Advisory Panel" program to overcome hurdles to marketing approval and to plan for post-market considerations

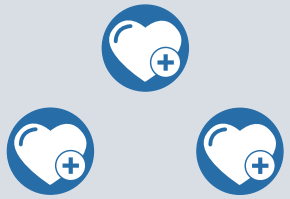
- Support healthcare infrastructure to support access of a diverse community to the rapidly maturing regenerative medicine landscape
  - Alpha Clinics expansion (academic medical centers)
  - Set up Community Care Centers of Excellence that are responsive to the needs of the given community

- Overcome manufacturing hurdles for regenerative medicine therapies by building a public-private manufacturing partnership network
  - By improving efficiencies, there is an opportunity to bring down cost of development/production

CIRM | Deliver Real World Solutions

Real Life™

Expand Alpha Clinics and create Community Care Centers of Excellence to support inclusive clinical research and treatments; serve as “real world settings” to study and implement novel care delivery



Expand Alpha Clinics Network *

Enable Innovative Clinical Research

Increase Patient Access to Therapies

Train Future Workforce

Equip Community Care* Centers to serve the needs of the community as identified by the community

*stipulated in Prop 14

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CIRM | Provide Opportunity for All

Real Life™

The Regenerative Medicine field is advancing and maturing rapidly

Focused and deliberate actions are needed to ensure diverse workforce and patient participation in the discovery, development and delivery of therapies

- **Build a diverse and highly skilled workforce** to support the growing regenerative medicine economy in California*
- **Deliver a roadmap for access and affordability** of regenerative medicine for all California patients*

*Education programs and AAWG are stipulated in Prop 14

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Payment Models Regenerative Medicine Products:

2017 Kymriah® and Yescarta®: cell-gene therapy, CAR-T for leukemia and lymphoma
ICER analysis concluded that proposed prices aligned with clinical benefit
UK NICE used ICER's evidence-based analysis. France and the UK, reimbursement is on the condition of collecting additional data (at the cohort level) and subject to future reassessments; elsewhere, rebates (Germany) or staged payments (Italy and Spain) are linked to individual patient outcomes.

2017 Luxturna®: prescription gene therapy for people with inherited retinal disease
Rebates provided if patient outcomes fail to meet a specified threshold for both short-term efficacy (30-90 days) and longer-term durability (30 months) measures that are unique to this one-time gene therapy.

2019 Zolgensma®: gene therapy for children under 2 years old with spinal muscular atrophy.
Payments made over time based on clinical outcomes.

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The growing need for VBAs

From presentation by Anand Kapur, HBSc, MBA
TwoLabsPharma Services

FDA Approved Products		
Product	**Type of therapy**	**Price**
Kymriah	Cell therapy	$475,000 / treatment
Yescarta	Cell therapy	$373,000 / treatment
Luxturna	Gene therapy	$425,000 / eye $850,000 / treatment
Spinraza	Gene therapy	$750,000 year 1 $375,000 subsequent years
Zolgensma	Gene therapy	$2.1 million

Sources: PriceRx, BioMedTracker

CIRM has tracked conversations led by:

- Alliance for Regenerative Medicine (ARM): Federal policy development Government Relations & Market Access and Value Committees – CMS discussions re. “Medicare Best Price” and “Anti-kick back” with respect to value-based models
- New Drug Development Paradigms Initiative (NEWDIGS MIT): Policy research and development model development for value / demonstration projects
- Duke Margolis Center for Health Policy: Policy research and development- Real World Evidence & model development
- Bipartisan Policy Center: Patient Centered Advocacy – 21st Century Cures Act 2016
- ICER and NICE: Value assessment frameworks for new products- tracking evaluation of CAR-T products and recent Sickle Cell treatments

Real Life™

World-Class Science | Real World Solutions

Opportunity for All